SAREPTA THERAPEUTICS

Patients can't wait for the next breakthrough in medical research.

So neither will we.

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. Every day is another 24 hours to stand up for patients, advance technology, challenge convention and **drag tomorrow into today**.

BENJAMIN
Living with Duchenne muscular dystrophy

FULL-TIME EMPLOYEES	MARKETED PRODUCTS	REVENUES FOR YEAR ENDING 12/31/2024	2024 R&D INVESTMENT
~850	4	$1.9B	$804M representing **48%** of operating expense

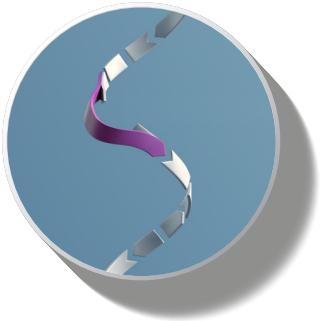
Data on file. Sarepta Therapeutics, Inc. 2025

Advancing an Industry-Leading Genetic Medicine Pipeline

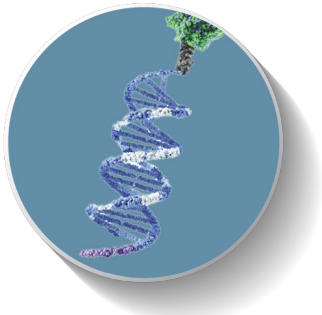
Sarepta holds leadership positions in Duchenne muscular dystrophy (Duchenne) and is building a robust portfolio of programs across muscle, central nervous system, and cardiac diseases.

Our pipeline includes numerous programs across three scientific platforms and multiple therapeutic areas.

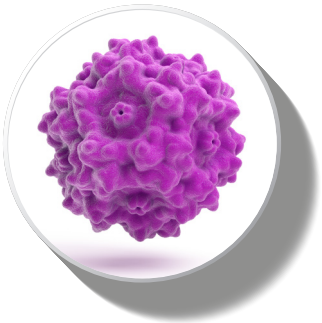
THREE SCIENTIFIC PLATFORMS



RNA PLATFORM
Proprietary technology designed to "skip" over a genetic mutation and enable the body to make a shortened version of a protein[1]



siRNA PLATFORM
Proprietary technology designed to "knockdown" or suppress overexpression of gene mutations[2]



GENE THERAPY
Delivering a new copy of a missing or malfunctioning gene with a goal of targeting the underlying biological defect that causes a certain disease

[1]RNA platform. Sarepta Therapeutics, Inc. Accessed July 17, 2024. https://www.sarepta.com/science/rna-platform
[2]Science & Innovation. Arrowhead Pharmaceuticals. Accessed October 3, 2025. https://arrowheadpharma.com/science-and-innovation/

OUR PIPELINE

Updated August 2025

Program Name	Discovery/Preclinical	Clinical
siRNA		
SRP-1001 (ARO-DUX4)	Facioscapulohumeral muscular dystrophy, Type 1 (FSHD1)	
SRP-1003 (ARO-DM1)	Myotonic dystrophy, Type 1 (DM1)	
SRP-1004 (ARO-ATXN2)	Spinocerebellar ataxia type 2 (SCA2)	
SRP-1002 (ARO-MMP7)	Idiopathic pulmonary fibrosis (IPF)	
Other Targets[3]	Multiple	
Gene Therapy		
SRP-9003 (bidridistrogene xeboparvovec)	LGMD2E/R4 β-sarcoglycan	

[3]Other siRNA indications include Huntington's disease, SCA1 and SCA3

Learn more about our pipeline at sarepta.com/pipeline.

MARKETED PRODUCTS

Sarepta is a fully integrated biopharmaceutical company that is committed to delivering medicines to treat rare, genetic-based diseases, including Duchenne. Our Duchenne portfolio includes four treatments:

3 RNA exon-skipping therapies

1 gene therapy

**Candidates received accelerated approval in the U.S., confirmatory studies are ongoing*

Learn more about our products at sarepta.com/products.

MAIN OFFICES

COMPANY HEADQUARTERS
Cambridge, MA, USA

RESEARCH AND MANUFACTURING FACILITIES
Andover, MA, USA
Bedford, MA, USA

GENETIC THERAPIES CENTER OF EXCELLENCE
Columbus, OH, USA

INTERNATIONAL OFFICES
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SAREPTA THERAPEUTICS

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